**Application Number**: T2-00004 **Scientific Score**: 85

*Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.*

**Title**: [redacted] CIRM Stem Cell Training Grant (Type II)

**Proposal Abstract as Submitted by Applicant**

The [redacted] proposes a CIRM type II program to train pre-doctoral PhD students and post-doctoral scientists. Currently, [redacted] faculty direct a large stem cell research and teaching enterprise that comprises over 100 biologists, chemists, engineers and clinicians with extensive expertise in stem cell biology and in allied disciplines dedicated to stem cell-based therapies for cardiovascular, neurodegenerative, hematopoietic and metabolic disorders. Additionally, the Institute has made substantial technology, recruitment and infrastructure investments as part of its commitment to stem cell biology. Our training curriculum will incorporate 1) an intensive hESC training course that has been run for two years and offers practical, hands-on instruction, 2) additional courses in stem cell, development, animal models of disease, bioinformatics and chemical biology, 3) training in ethical and legal implications of stem cells, and 4) laboratory research. Courses will be open to other CIRM program trainees in the [redacted] area and students will benefit from our inter-institutional research and training collaborations. PhD degrees will be granted through our existing training partnership with [redacted] and through an independent [redacted] PhD program.

**Benefit of this Program to California**

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

**Summary of Review**

This is an impressive proposal for a type II training program that will focus on the development and use of human embryonic stem cells (hESC). A secondary focus is on stem cells that reside in differentiated tissues. The proposal augments an existing training program in stem cell biology that is supported by an outstanding faculty with expertise in stem cell research, an NIH-funded human ESC training course, and an NIH-funded infrastructure grant. The training program will be conducted in a collaborative way among four biomedical institutions. The quality of the proposed training program and integration of its components is exceptionally high. Training will be comprehensive, spanning from the classroom to laboratory and active research settings. The primary focus will be on the derivation and use of new hESC lines toward the development of advanced research technologies and medical applications. This aim represents one of the strongest aspects of the application. The program director is a professor with appointments at this institution and an affiliated medical school. The director is an exceptionally gifted leader, administrator, mentor, trainer, and researcher. With many years of leadership, the director has previously participated in curriculum design,

admissions, and thesis advisory committees at Harvard Medical School. A 6-member executive committee chosen for their expertise in stem cell biology will select trainees, evaluate and select mentors, and set the program's agenda. A faculty of 26 well-known investigators is listed and bio-sketches indicate high productivity and publications in first-rate journals. All key research faculty members have successfully obtained NIH RO1 funding to support their work in stem cell biology and all have mentoring experience. The institution plans to launch an independent graduate program this year. The applicant pool is expected to be very good given the cross-pollination to its affiliated university's highly regarded graduate programs. However, the applying institution's graduate program must stand on its own, so requested pre-doctoral slots should await demonstration that this new program is adequate for training in stem cell biology.

**Overall Strengths and Weaknesses**

This strong application augments an existing program in stem cell biology with a comprehensive training program. The quality of the program director, training faculty, and facilities is very high and is strengthened further by the collaborative effort of four participating institutions. The primary focus on hESC is a great strength of the program. However, the integration of basic and medical applications is not discussed and represents one weakness of this proposal. A second concern is the proposed in-house graduate program which is about to be launched. Because of the newness of this endeavor, there is no history of successful training.

**Recommendations**

Highly meritorious and recommended for funding. CIRM recommends a reduction of pre-doctoral trainee slots and encourages the applicant to submit an application at a later date for a supplement to fund these positions when concerns have been addressed.

| | Pre | Post | Clinical | Total |
|---|---|---|---|---|
| Fellows Requested: | 5 | 5 | 0 | 10 |
| Fellows Recommended: | 0 | 5 | 0 | 5 |

| | Year 1 | Total |
|---|---|---|
| Budget Requested: | $ 717,052 | $ 2,254,313 |
| Budget Recommended: | $ 410, 300 | $ 1,265,900 |